Opening Statement of the Honorable Joseph R. Pitts
Subcommittee on Health
Hearing on "21st Century Cures: Incorporating the Patient Perspective"
July 11, 2014

(As Prepared for Delivery)

Today's hearing provides us with an opportunity to examine perhaps one of the most important aspects of the 21st Century Cures Initiative: what does medical innovation or faster cures mean for patients. Keeping our work centered on the patient and understanding the patient perspective will bring much needed focus on results for patients who may lack adequate treatment options. Remember, there are only effective treatments for 500 of the 7,000 known diseases impacting patients today.

While FDA has developed an enhanced structured approach to benefit-risk assessment in regulatory decision-making for human drug, device and biologic products, the Committee recognizes the value of considering patients in decision-making about therapy development and access. Assessment of a drug or device's benefits and risks includes an analysis of the severity of the condition treated and the current treatment options available and getting the patient's unique perspective should be a part of that assessment.

One of our witnesses today, Pat Furlong of Parent Project Muscular Dystrophy – PPMD - will explain how this organization was founded to create opportunities for families waiting for therapies to stop Duchene muscular dystrophy from claiming young lives. To quote Pat Furlong, "Patient focused drug development acknowledges the need to gather input from patients and their caregivers to create a more complete assessment of the benefit-risk equation, encouraging predictability and increased flexibility within the review process. The clock is ticking for patients who need and deserve access to promising therapies." I would like to applaud her tireless work drafting guidance PPMD recently released that actually quantifies patient priorities and preferences. This guidance will serve the Duchene community and every other patient community because it provides a path for other patient groups to follow. This was an enormous undertaking, and I am confident it will make a substantial contribution to the entire medical community.

I want to welcome our witnesses today and look forward to learning more about the assessment of benefits and risks central to medical product development, regulations, and healthcare decision-making, and the tradeoffs between desired benefits and tolerable risks.

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